



Personalized Cell Therapy for Diabetes

Grant Award Details

Personalized Cell Therapy for Diabetes

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-08561-B

Project Objective: To optimize manufacturing, safety & efficacy testing of cell therapy product, and complete a Pre-

IND meeting with the FDA.

Investigator:

Name: Peter Butler

Institution: University of California, Los

Angeles

Type: PI

Disease Focus: Diabetes, Metabolic Disorders

Human Stem Cell Use: iPS Cell

Cell Line Generation: iPS Cell

Award Value: \$4,230,186

Status: Active

Grant Application Details

Application Title: Personalized Cell Therapy for Diabetes

Public Abstract:

Translational Candidate

Preclinical studies will develop patient specific stem cell-derived islets that secrete insulin & other islet hormones for regulation of blood sugar

Area of Impact

Genetically matched stem cell derived islets could provide treatment for diabetes without the need for immunosuppression or implantable devices.

Mechanism of Action

The stem cell-derived islets contain insulin-producing pancreatic β cells. These differentiated cells will be transplanted into patients who suffer from diabetes in order to replace the β cells that are missing or dysfunctional in the pancreatic islets of those patients. The stem cells used to generate these replacement islets will be genetically matched to the patient, enabling transplantation without long-term immunosuppression.

Unmet Medical Need

Millions suffer from Type 1 & Type 2 diabetes, which significantly impact quality of life and lead to serious complications. This proposal develops an alternative therapy with potential to transform the lives of those patients. First target is patients with insulin dependent, non-autoimmune diabetes

Project Objective

Pre-IND meeting with the FDA

Major Proposed Activities

- Production of the starting stem cell material from patients
- Optimize manufacturing of differentiated cell therapy product
- Preclinical safety and efficacy studies of the product

Statement of Benefit to California:

Many CA citizens are impacted by diabetes, including particularly high numbers with non-autoimmune, insulin dependent diabetes. This translational research aims to bring academic discoveries from lab of Doug Melton to the clinic, through generation of patient specific stem cell derived islets for transplant. Success in this program could have have immediate benefit to the California patients involved and would also lead the way to a new cell therapy broadly applicable to people with diabetes.

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